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Abeona Therapeutics Announces License for Next Generation AAV Delivery Vector for Fanconi Anemia and Rare Blood Disease Platform

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Abeona Therapeutics, Inc. (NASDAQ: ABEO)

- License enables lead CRISPR/Cas9 program for ABO-301 (AAV-FANCC) for Fanconi anemia and rare blood disorder platform
- CRISPR/Cas9 mediated FANCC correction has demonstrated efficacy in cells from Fanconi anemia patients
- Preclinical studies have demonstrated promising efficacy for treating Fanconi anemia disorder, a rare, fatal autosomal recessive disease

Abeona Therapeutics, Inc. (NASDAQ: ABEO), a biopharmaceutical company focused on developing and delivering gene therapy and plasma-based products for severe and life-threatening rare diseases, today announced a license agreement with Stanford University for an AAV (adeno-associated virus) delivery vector for the treatment of Fanconi anemia (FA) and rare blood disease platform. This license augments a previously announced license agreement with the University of Minnesota for ABO-301 (AAV-FANCC) to treat patients with Fanconi anemia (FA) disorder and other rare blood diseases.

"Effective product development in gene therapy requires choosing the right delivery vehicle because each virus has a unique transduction profile that determines its ability to transfer genetic material from one organism to another," stated Steven Rouhandeh, Executive Chairman. "This licensing transaction complements our near-clinical stage programs in Sanfilippo syndromes (MPS IIIA and MPS IIIB), and juvenile Batten disease, as well as further expands our commitment to building a leadership position in the gene therapy space, with a focus on developing therapies for patients with rare diseases."

The major function of bone marrow is to produce new blood cells. In FA, a DNA mutation renders the FANCC gene nonfunctional. Loss of FANCC causes patient skeletal abnormalities and leads to bone marrow failure. Fanconi anemia patients also have much higher rates of hematological diseases, such as acute myeloid leukemia (AML) or tumors of the head, neck, skin, gastrointestinal system, or genital tract. The likelihood of developing one of these cancers in people with Fanconi anemia is between 10 and 30 percent. Aside from bone marrow transplantation (BMT) there are no specific treatments known that can halt or reverse the symptoms of FA. Repairing fibroblast cells in FA patients with a functional FANCC gene is the focus of our AAV-based gene therapy approach.

"The FANCC gene encodes a protein that functions as part of the pathway responsible for genome surveillance and repair of DNA damage. Hematopoietic progenitors are an ideal population for correction for therapeutic use. Our strategies encompass first and second generation therapies to correct hematopoietic cells," noted Tim Miller, Ph.D, President & CEO. "We are leveraging the unique capabilities of the CRISPR-Cas9 gene editing platform technology to build a robust product pipeline to address the unmet needs of patients that may have one of a variety of blood diseases, which include Beta-thalassemia, Fabry disease, as well as inherited forms of neutropenia, thrombocytopenia, and anemias."

About ABO-301 (AAV FANCC): ABO-301 is an AAV-based gene therapy which has shown promising preclinical efficacy in delivery of a normal copy of the defective gene to cells of the hematopoietic or blood system with the aim of reversing the effects of the genetic errors that cause Fanconi anemia.

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Using a novel CRISPR (clustered, regularly interspaced short palindromic repeats)-Cas9 (CRISPR associated protein 9) system, researchers used a protein-RNA complex composed of an enzyme known as Cas9 bound to a guide RNA molecule that has been designed to recognize a particular DNA sequence. The RNA molecules guide the Cas9 complex to the location in the genome that requires repair. CRISPR-Cas9 uniquely enables surgically efficient knock-out, knock-down or selective editing of defective genes in the context of their natural promoters, unlocking the potential to treat both recessive and dominant forms of genetic diseases. Most importantly, this approach has the potential to allow safer, more precise gene modification for a wide range of rare blood diseases.

About Abeona: Abeona Therapeutics, Inc. develops and delivers gene therapy and plasma-based products for severe and life-threatening rare diseases. Abeona's lead programs are AB0-101 (AAV9 NAGLU) and ABO-102 (scAAV9 SGSH), adeno-associated virus (AAV)-based gene therapies for Sanfilippo syndrome (MPS IIIB and IIIA) in collaboration with patient advocate groups, researchers and clinicians, anticipated to commence clinical trials in 2015. We are also developing ABO-201 (scAAV9 CLN3) gene therapy for juvenile Batten disease (JBD); and ABO-301 (AAV FANCC) for Fanconi anemia (FA) disorder using a novel CRISPR/Cas9-based gene editing approach to gene therapy program for rare blood diseases. In addition, we are also developing rare plasma protein therapies including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD using our proprietary SDF™ (Salt Diafiltration) ethanol-free process. For more information, visit www.abeonatherapeutics.com.

This press release contains certain statements that are forward-looking within the meaning of Section 27a of the Securities Act of 1933, as amended, and that involve risks and uncertainties. These statements include, without limitation, our plans for the use of proceeds

of the financing, our plans to begin enrolling patients in clinical trials for the treatment of Sanfilippo syndrome, development and internationalization of other clinical programs, management plans for the Company, the anticipated closing of the transaction, and general business outlook. These statements are subject to numerous risks and uncertainties, including but not limited to continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the impact of competition; the ability to develop our products and technologies; the ability to achieve or obtain necessary regulatory approvals; the impact of changes in the financial markets and global economic conditions; and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and other reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligations to make any revisions to the forward-looking statements contained in this release or to update them to reflect events or circumstances occurring after the date of this release, whether as a result of new information, future developments or otherwise.

Company and Media Contact:
Andre'a Lucca
Vice President of Communications
Abeona Therapeutics, Inc.
+1 (212)-786-6208
alucca@abeonatherapeutics.com

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